Innovation Pathway

Program at FDA

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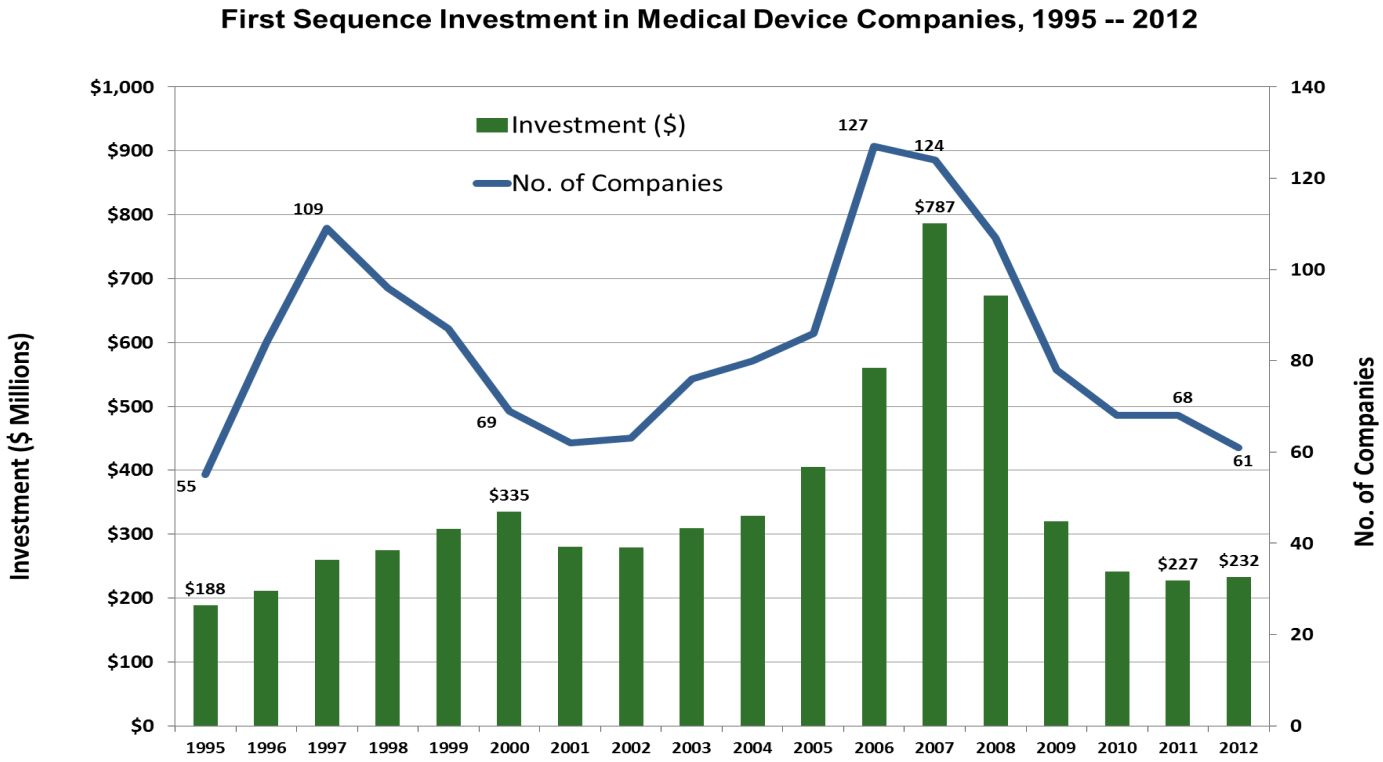
Medical Devices for Pediatric Patients Affected by Rare Diseases January 8, 2014

The Crisis in Medtech Innovation

Start-up Investment Has Fallen >70% Since 2007



Source: PWC/NVCA Moneytree Report, 2Q2-12
Courtesy: Ross Jaffe

Key Contributors

Factors cited as having the highest impact on decisions to move medical device investment outside of U.S.*

Regulatory Challenges – 38%
Reimbursement Concerns – 18%
Clinical Trial Issues – 14%

**from National Venture Capital Association/Medical Innovation & Competitiveness Coalition survey of 259 NVCA member firms investing in the healthcare sectors; 60% (156 firms responding) October, 2011*

What is the Current Landscape of Medical Device Development?

- FDA acknowledges real or perceived problems:
  - Initial clinical testing of novel devices has moved to non-US sites
  - Device innovation may follow overseas
  - Devices are being exclusively developed for non-US markets
  - FDA's requirements can be an impediment to early clinical testing of new devices
- Growing concern regarding the time lag in the availability of beneficial medical devices for US patients

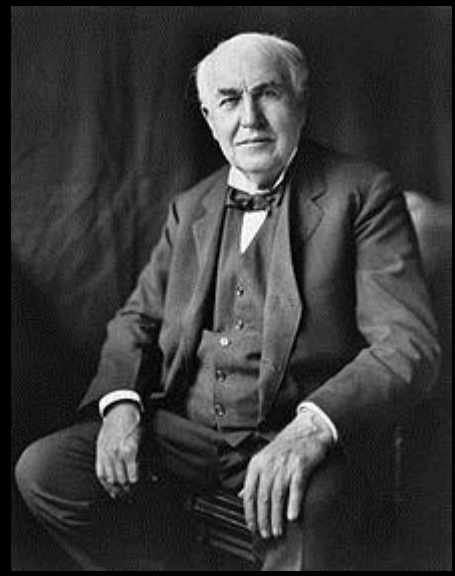
INNOVATION PATHWAY GUIDING PRINCIPLES

1) When the goals are understood, the methods to achieve them can be improvised

2) We value improvisation, experimentation, risk-taking, and problem-solving over process-following and standardization (uniformity of approach)

3) Okay to fail

Edison: "Hell, we ain't got no rules around here. We're trying to accomplish something!"



Innovation Program Goals

1. Shorten the time from concept to commercialization
   - Earlier contact
   - Stronger Benefit/Risk:
     - Patient Perspectives
     - Disease considerations, alternative therapies, unmet needs
     - Mitigation strategies
   - Support Early Feasibility/FIH studies

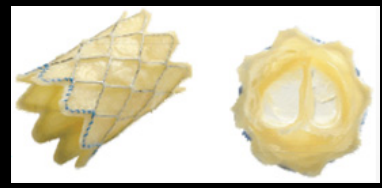
2. Transform the user experience
   - One Team: collaboration is the key
   - Create a Map of Regulatory Pathway
   - IT tools (“Sales Force” platform)

3. Make decisions that create forward momentum

Words into Deeds

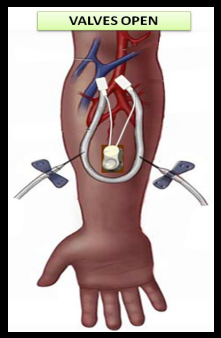
The core principles in meeting these goals are the application of specific guidelines throughout the regulatory decision-making process:

- Benefit/Risk Principles
  - Pediatric-sized heart valves (PMA supplement)
  - Berlin Heart – unmet need
  - PumpKin Project (VAD/ECMO) HUD devices
- Early Feasibility Studies
  - Medtronic Transcatheter pulmonary valve
- The Innovation Pathway
  - End stage renal disease challenge
  - Entrepreneurs-in-Residence Program



END-STAGE RENAL DISEASE INNOVATION PATHWAY CHALLENGE

- 32 Device applicants; 3 selected
- Collaboration Phase – Summer 2012

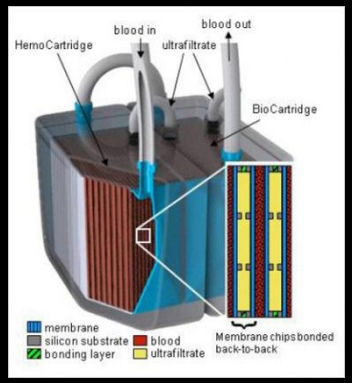


HemoAccess Valve System

- A mechanical valve system allowing blood flow into AV graft only during dialysis

Wearable Artificial Kidney

- A miniaturized wearable dialysis machine to provide hemodialysis



Implantable Bio-Artificial Kidney

(Univ. California, San Francisco)

- Filters toxins from blood, and provide other biological functions giving patients 24/7 dialysis

INNOVATION PATHWAY ENTREPRENEURS IN RESIDENCE (EIR) PROGRAM

- Overview: Time-limited recruitment of world-class innovators entrepreneurs and visionaries from a variety of medical-device related fields to join highly-qualified FDA staff in the development of solutions in areas that impact innovation

- Goal: Transformational change by combining internal and external talent applying Innovation Pathway principles

  - Streamlining the Clinical Trials Enterprise
  - Streamlining pathway from approval to reimbursement
  - Striking the right balance between pre- and post-market evidentiary requirements

Thank You